



Office de la Propriété
Intellectuelle
du Canada

Un organisme
d'Industrie Canada

Canadian
Intellectual Property
Office

An agency of
Industry Canada

CA 2507606 A1 2004/06/10

(21) 2 507 606

(12) DEMANDE DE BREVET CANADIEN
CANADIAN PATENT APPLICATION

(13) A1

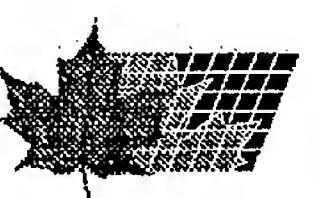
(86) Date de dépôt PCT/PCT Filing Date: 2003/11/26
(87) Date publication PCT/PCT Publication Date: 2004/06/10
(85) Entrée phase nationale/National Entry: 2005/05/26
(86) N° demande PCT/PCT Application No.: US 2003/037650
(87) N° publication PCT/PCT Publication No.: 2004/047872
(30) Priorités/Priorities: 2002/11/26 (60/429,387) US;
2003/02/03 (60/444,614) US

(51) Cl.Int.⁷/Int.Cl.⁷ A61K 31/713
(71) Demandeur/Applicant:
MEDTRONIC, INC., US
(72) Inventeur/Inventor:
KAEMMERER, WILLIAM F., US
(74) Agent: SMART & BIGGAR

(54) Titre : TRAITEMENT DE MALADIE NEURODEGENERATIVE PAR ADMINISTRATION INTRACRANIENNE D'ARNI
(54) Title: TREATMENT OF NEURODEGENERATIVE DISEASE THROUGH INTRACRANIAL DELIVERY OF SHORT
INTERFERING RNA (SIRNA)

(57) Abrégé/Abstract:

The present invention provides devices, small interfering RNA, and methods for treating a neurodegenerative disorder comprising the steps of surgically implanting a catheter so that a discharge portion of the catheter lies adjacent to a predetermined infusion site in a brain, and discharging through the discharge portion of the catheter a predetermined dosage of at least one substance capable of inhibiting production of at least one neurodegenerative protein. The present invention also provides valuable small interfering RNA vectors, and methods for treating neurodegenerative disorders such as Alzheimer's disease, Parkinson's disease, Huntington's disease, Spinocerebellar Ataxia Type I, Type 2, Type 3, and/or dentatorubral-pallidoluysian atrophy.



(12) INTERNATIONAL APPLICATION PUBLISHED UNDER THE PATENT COOPERATION TREATY (PCT)

(19) World Intellectual Property
Organization
International Bureau



(43) International Publication Date
10 June 2004 (10.06.2004)

PCT

(10) International Publication Number
WO 2004/047872 A3

(51) International Patent Classification⁷: **A61K 31/713**

(84) Designated States (*regional*): ARIPO patent (BW, GH, GM, KE, LS, MW, MZ, SD, SL, SZ, TZ, UG, ZM, ZW), Eurasian patent (AM, AZ, BY, KG, KZ, MD, RU, TJ, TM), European patent (AT, BE, BG, CH, CY, CZ, DE, DK, EE, ES, FI, FR, GB, GR, HU, IE, IT, LU, MC, NL, PT, RO, SE, SI, SK, TR), OAPI patent (BF, BJ, CF, CG, CI, CM, GA, GN, GQ, GW, ML, MR, NE, SN, TD, TG).

(21) International Application Number:
PCT/US2003/037650

(22) International Filing Date:
26 November 2003 (26.11.2003)

Declaration under Rule 4.17:

— *as to applicant's entitlement to apply for and be granted a patent (Rule 4.17(ii)) for the following designations AE, AG, AL, AM, AT, AU, AZ, BA, BB, BG, BR, BW, BY, BZ, CA, CH, CN, CO, CR, CU, CZ, DE, DK, DM, DZ, EC, EE, EG, ES, FI, GB, GD, GE, GH, GM, HR, HU, ID, IL, IN, IS, JP, KE, KG, KP, KR, KZ, LC, LK, LR, LS, LT, LU, LV, MA, MD, MG, MK, MN, MW, MX, MZ, NI, NO, NZ, OM, PG, PH, PL, PT, RO, RU, SC, SD, SE, SG, SK, SL, SY, TJ, TM, TN, TR, TT, TZ, UA, UG, UZ, VC, VN, YU, ZA, ZM, ZW, ARIPO patent (BW, GH, GM, KE, LS, MW, MZ, SD, SL, SZ, TZ, UG, ZM, ZW), Eurasian patent (AM, AZ, BY, KG, KZ, MD, RU, TJ, TM), European patent (AT, BE, BG, CH, CY, CZ, DE, DK, EE, ES, FI, FR, GB, GR, HU, IE, IT, LU, MC, NL, PT, RO, SE, SI, SK, TR), OAPI patent (BF, BJ, CF, CG, CI, CM, GA, GN, GQ, GW, ML, MR, NE, SN, TD, TG)*

(25) Filing Language: English

(26) Publication Language: English

(30) Priority Data:
60/429,387 26 November 2002 (26.11.2002) US
60/444,614 3 February 2003 (03.02.2003) US

(71) Applicant: MEDTRONIC, INC. [US/US]; MS LC340, 710 Medtronic Parkway NE, Minneapolis, MN 55432 (US).

(72) Inventor: KAEMMERER, William, E.; 4900 Trillium Lane, Edina, MN 55435 (US).

(74) Agents: COLLIER, Kenneth, J. et al.; MC LC340, 710 Medtronic Parkway, Minneapolis, MN 55432 (US).

(81) Designated States (*national*): AE, AG, AL, AM, AT, AU, AZ, BA, BB, BG, BR, BW, BY, BZ, CA, CH, CN, CO, CR, CU, CZ, DE, DK, DM, DZ, EC, EE, EG, ES, FI, GB, GD, GE, GH, GM, HR, HU, ID, IL, IN, IS, JP, KE, KG, KP, KR, KZ, LC, LK, LR, LS, LT, LU, LV, MA, MD, MG, MK, MN, MW, MX, MZ, NI, NO, NZ, OM, PG, PH, PL, PT, RO, RU, SC, SD, SE, SG, SK, SL, SY, TJ, TM, TN, TR, TT, TZ, UA, UG, UZ, VC, VN, YU, ZA, ZM, ZW.

Published:

— *with international search report*

(88) Date of publication of the international search report:
3 February 2005

For two-letter codes and other abbreviations, refer to the "Guidance Notes on Codes and Abbreviations" appearing at the beginning of each regular issue of the PCT Gazette.

WO 2004/047872 A3

(54) Title: TREATMENT OF NEURODEGENERATIVE DISEASE THROUGH INTRACRANIAL DELIVERY OF SHORT INTERFERING RNA (SIRNA)

(57) Abstract: The present invention provides devices, small interfering RNA, and methods for treating a neurodegenerative disorder comprising the steps of surgically implanting a catheter so that a discharge portion of the catheter lies adjacent to a predetermined infusion site in a brain, and discharging through the discharge portion of the catheter a predetermined dosage of at least one substance capable of inhibiting production of at least one neurodegenerative protein. The present invention also provides valuable small interfering RNA vectors, and methods for treating neurodegenerative disorders such as Alzheimer's disease, Parkinson's disease, Huntington's disease, Spinocerebellar Ataxia Type 1, Type 2, Type 3, and/or dentatorubral-pallidoluysian atrophy.